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(FILE 'HOME' ENTERED AT 17:44:44 ON 16 MAY 2007) FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 17:45:12 ON 16 MAY 2007 L1 16134 S (LYSOSOMAL OR FABRY) (3A) (DISEASE OR DISORDER) L2 178921 S LYSOSOMAL (4A) HYDROLASE OR GALACTOSIDASE OR SULFATASE OR NEURA L3 1476 S (TREAT? OR INHIBIT? OR AMELIORAT? OR DECREAS? OR REDUC?) (8A) L L4566 S L2 AND L3 407 S L2(P)L3 L5 27875 S (GENE OR DNA OR CDNA OR POLYNUCLEOTIDE OR NUCLEIC (W) ACID) (8A) L6 L7 41 S L3(P)L6 41 S L4 AND L7 L8 L9 28 DUP REM L8 (13 DUPLICATES REMOVED) => d au ti so pi ·1-28 19 L9 ANSWER 1 OF 28 MEDLINE on STN DUPLICATE 1 ΑU Santamaria R; Blanco M; Chabas A; Grinberg D; Vilageliu L TТ Identification of 14 novel GLB1 mutations, including five deletions, in 19 patients with GM1 gangliosidosis from South America. SO Clinical genetics, (2007 Mar) Vol. 71, No. 3, pp. 273-9. Journal code: 0253664. ISSN: 0009-9163. L9 ANSWER 2 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN ΑU Sirrs, Sandra M.; Clarke, Joe T. R. TI Agalsidase alfa therapy for Fabry disease SO Expert Review of Endocrinology & Metabolism (2007), 2(2), 147-154 CODEN: EREMBI; ISSN: 1744-6651 L9 ANSWER 3 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN ΑU Marino, Silvia; Borsini, Walter; Buchner, Susanna; Mortilla, Marzia; Stromillo, Maria L.; Battaglini, Marco; Giorgio, Antonio; Bramanti, Placido; Federico, Antonio; De Stefano, Nicola TI Diffuse structural and metabolic brain changes in Fabry disease SO Journal of Neurology (2006), 253(4), 434-440 CODEN: JNRYA9; ISSN: 0340-5354 L9 ANSWER 4 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN IN Fan, Jian-Qiang TI Combination therapy for treating protein deficiencies PCT Int. Appl., 40 pp. SO CODEN: PIXXD2 PATENT NO. KIND DATE APPLICATION NO. ---------PΙ WO 2004074450 A2 20040902 WO 2004-US4909 20040218 WO 2004074450 **A8** 20041104 WO 2004074450 **A3** 20050825 AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI RW: BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG US 2004219132 20041104 US 2004-781356 A1 20040217 CA 2516304 Α1 20040902 CA 2004-2516304 20040218 EP 1594514 20051116 EP 2004-712428 A2 20040218 AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT,

IE, SI, LT, LV, FI, RO, MK, CY, AL; TR, BG, CZ, EE, HU, SK

BR 2004-7648

20040218

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IN 2005DN04230	A	20070427	IN 2005	5-DN4230	20050919

- L9 ANSWER 5 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Cheng, Seng H.; Meeker, David
- TI Combined enzyme replacement, gene therapy and small molecule therapy for lysosomal storage diseases
- SO U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526. CODEN: USXXCO

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
ΡI	US 2004204379	<b>A1</b>	20041014	US 2004-758773	20040116
	US 2002095135	A1	20020718	US 2001-884526	20010619

- L9 ANSWER 6 OF 28 SCISEARCH COPYRIGHT (c) 2007 The Thomson Corporation on STN
- AU Masson C (Reprint); Cisse D; Simon V; Insalaco P; Audran M
- TI Fabry disease: a review
- SO JOINT BONE SPINE, (SEP 2004) Vol. 71, No. 5, pp. 381-383. ISSN: 1297-319X.
- L9 ANSWER 7 OF 28 MEDLINE on STN DUPLICATE 2
- AU Ziegler Robin J; Lonning Scott M; Armentano Donna; Li Chester; Souza David W; Cherry Maribeth; Ford Christine; Barbon Christine M; Desnick Robert J; Gao Guangping; Wilson James M; Peluso Richard; Godwin Simon; Carter Barrie J; Gregory Richard J; Wadsworth Samuel C; Cheng Seng H
- TI AAV2 vector harboring a liver-restricted promoter facilitates sustained expression of therapeutic levels of alpha-galactosidase A and the induction of immune tolerance in Fabry mice.
- SO Molecular therapy: the journal of the American Society of Gene Therapy, (2004 Feb) Vol. 9, No. 2, pp. 231-40.

  Journal code: 100890581. ISSN: 1525-0016.
- L9 ANSWER 8 OF 28 MEDLINE on STN DUPLICATE 3
- AU Palla A; Widmer U; Straumann D
- TI Head-impulse testing in Fabry disease--vestibular function in male and female patients.
- SO Acta paediatrica (Oslo, Norway: 1992). Supplement, (2003 Dec) Vol. 92, No. 443, pp. 38-42; discussion 27.

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- L9 ANSWER 9 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Garger, Stephen J.; Turpen, Thomas H.; Kumagai, Monto H.
- TI Production of human glucocerebrosidase and  $\alpha$  galactosidase in transgenic tobacco plants and their potential use in enzyme replacement therapy and treatment of lysosomal storage diseases
- SO U.S. Pat. Appl. Publ., 88 pp., Cont.-in-part of U.S. Ser. No. 626,127. CODEN: USXXCO

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
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	AU 9918610	A	19990506	AU 1999-18610	19990305
	AU 741443	B2	20011129		
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	US 2003106095	A1	20030605	US 2002-103327	20020320
	US 6890748	B2	20050510		
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	US 2004023281	A1	20040205	US 2003-602220	20030623
	US 2004234516	A1	20041125	US 2004-851388	20040521
	US 2005125859	A1	20050609	US 2004-984389	20041108

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L9 ANSWER 10 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
IN Selden, Richard F.; Borowski, Marianne; Kinoshita, Carol M.; Treco,
Douglas A.; Williams, Melanie D.; Schuetz, Thomas J.; Daniel, Peter F.
TI Purification of recombinant α- galactosidase A and its
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glycosylation modification for treatment of Fabry disease and related therapy by targeted gene activation

SO U.S., 39 pp., Cont.-in-part of U. S. Ser. No. 928,881.

CODEN: USXXAM

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	AU	2004	2425	50		A1		2005	0127		AU 2	004-	2425	50		20	0041	231

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AU Ashley Grace A; Desnick Robert J; Gordon Ronald E; Gordon Jon W

Journal code: 9501229. ISSN: 1081-5589.

TI High overexpression of the human alpha-galactosidase A gene driven by its promoter in transgenic mice: implications for the treatment of Fabry disease.

SO Journal of investigative medicine: the official publication of the American Federation for Clinical Research, (2002 May) Vol. 50, No. 3, pp. 185-92.

L9 ANSWER 12 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN

AU Germain, Dominique Paul

TI Fabry disease (α- galactosidase A deficiency): pathophysiology, clinical signs and genetics aspects

SO Journal de la Societe de Biologie (2002), 196(2), 161-173 . CODEN: JDSBFG; ISSN: 1295-0661

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     Takiyama, Nobuaki
TI
     Retroviral mediated gene transfer to human CD34+ hematopoietic cells: gene
     therapy of Gaucher and Fabry diseases
     Keio Igaku (2002), 79(1), T143-T152
SO
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     ANSWER 14 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
     Ziegler, Robin; Cheng, Seng; Marshall, John; Goldberg, Mark
IN
     Methods for treatment of lysosomal storage
ΤI
     diseases
     PCT Int. Appl., 25 pp.
SO
     CODEN: PIXXD2
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     WO 2001060377
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     WO 2001060377
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     US 2001031741
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                               20040610
                                          US 2003-632302
                                                                20030801
     ANSWER 15 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
L9
     Miyamura, Nobuhiro
IN
TI
     Protein and cDNA sequences of truncated human \alpha-
     galactosidase A and uses thereof in treatment of
     Fabry disease
so
     U.S., 51 pp.
     CODEN: USXXAM
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     PATENT NO.
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IN
     Davidson, Beverly L.; Jolly, Douglas J.; Sauter, Sybille L.; Ghodsi, Adbi;
     Stein, Colleen S.; Derksen, Todd A.; Dubensky, Thomas W., Jr.
     Recombinant gene delivery vectors for treating or preventing
TI
     lysosomal storage disorders
SO
     PCT Int. Appl., 52 pp.
     CODEN: PIXXD2
     PATENT NO.
                       KIND DATE
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    WO 2000073482
                       A1 20001207 WO 2000-US14582 20000526
PΙ
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     EP 1183384
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            IE, SI, LT, LV, FI, RO
    JP 2003523174
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                                          JP 2001-500794
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    US 6730297
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    US 2003223963
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                                          US 2003-356272
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    US 7034010
                       B2
                              20060425
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L9 ANSWER 17 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN

IN Selden, Richard F.; Borowski, Marianne; Kinoshita, Carol M.; Treco,

Douglas A.; Williams, Melanie D.; Schuetz, Thomas J.; Daniel, Peter F. TI Methods for modification of  $\alpha$ - galactosidase A glycosylation, for purification of enzyme, and for treatment of Fabry disease SO PCT Int. Appl., 92 pp. CODEN: PIXXD2 APPLICATION NO. PATENT NO. DATE ---------\_\_\_\_\_\_ WO 2000053730 PI A2 20000914 WO 2000-US6118 20000309 20010315 WO 2000053730 A3 W: AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CR, CU, CZ, DE, DK, DM, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZW RW: GH, GM, KE, LS, MW, SD, SL, SZ, TZ, UG, ZW, AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG 20021001 US 1999-266014 US 6458574 B1 19990311 CA 2365923 A1 20000914 CA 2000-2365923 20000309 AU 200035194 AU 2000-35194 Α 20000928 20000309 EP 1163349 A2 20011219 EP 2000-913825 20000309 AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO JP 2002538183 Т 20021112 JP 2000-603353 20000309 RU 2248213 C2 20050320 RU 2001-127533 20000309 NO 2001004415 Α 20011112 NO 2001-4415 20010911 AU 2001-93403 AU 762400 B2 20030626 20011123 AU 2003220717 A1 20030814 AU 2003-220717 20030722 AU 2004-242550 AU 2004242550 A1 20050127 20041231 L9 ANSWER 18 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN IN Yew, Nelson S.; Ziegler, Robin J.; Cheng, Seng H. TI Compositions and methods for treating lysosomal storage disease SO PCT Int. Appl., 54 pp. CODEN: PIXXD2 PATENT NO. DATE KIND APPLICATION NO. DATE ---------PΙ WO 2000009153 A1 20000224 WO 1998-US22886 W: AU, CA, IL, JP RW: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE CA 2305768 A1 20000224 CA 1998-2305768 19981029 AU 9912847 Α 20000306 AU 1999-12847 19981029 AU 734290 B2 20010607 US 6066626 20000523 Α US 1998-182245 19981029 EP 1027069 **A1** 20000816 EP 1998-956290 19981029 EP 1027069 B1 20060726 AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI, CY JP 2002522509 20020723 JP 2000-564655 19981029 EP 1658857 A1 20060524 EP 2006-833 19981029 AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI, CY AT 1998-956290 AT 333893 20060815 19981029 US 2003087868 US 2002-244700 **A1** 20030508 20020913 L9 ANSWER 19 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN Ohshima, Toshio; Schiffmann, Raphael; Murray, Gary J.; Kopp, Jeffrey; ΑU Quirk, Jane M.; Stahl, Stefanie; Chan, Chi-Chao; Zerfas, Patricia; Tao-Cheng, Jung-Hwa; Ward, J. M.; Brady, Roscoe O.; Kulkarni, Ashok B. TI Aging accentuates and bone marrow transplantation ameliorates metabolic defects in Fabry disease mice

- SO Proceedings of the National Academy of Sciences of the United States of America (1999), 96(11), 6423-6427
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- L9 ANSWER 20 OF 28 MEDLINE on STN
- AU Suzuki T; Fujino T; Sugasawa M; Kohara Y; Toyama K; Sato T; Yasuda T; Sugiyama M; Maeba T; Owada S; Ishida M
- TI A case of Fabry's disease with chronic renal failure.
- SO Nippon Jinzo Gakkai shi, (1999 Jun) Vol. 41, No. 4, pp. 448-53. Journal code: 7505731. ISSN: 0385-2385.
- L9 ANSWER 21 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Okumiya, Toshika; Kawamura, Osamu; Itoh, Kohji; Kase, Ryoichi; Ishii, Satoshi; Kamei, Sachiko; Sakuraba, Hitoshi
- TI Novel missense mutation (M72V) of  $\alpha$  galactosidase gene and its expression product in an atypical Fabry hemizygote
- SO Human Mutation (1998), (Suppl. 1), S213-S216 CODEN: HUMUE3; ISSN: 1059-7794
- L9 ANSWER 22 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Miyamura, Nobuhiro; Araki, Eiichi; Matsuda, Kohji; Yoshimura, Ryouhei; Furukawa, Noboru; Tsuruzoe, Kaku; Shirotani, Tetsuya; Kishikawa, Hideki; Yamaguchi, Kohei; Shichiri, Motoaki
- TI A carboxy-terminal truncation of human  $\alpha$  galactosidase A in a heterozygous female with Fabry disease and modification of the enzymic activity by the carboxy-terminal domain: increased, reduced, or absent enzyme activity depending on number of amino acid residues deleted
- SO Journal of Clinical Investigation (1996), 98(8), 1809-1817 CODEN: JCINAO; ISSN: 0021-9738
- L9 ANSWER 23 OF 28 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Desnick, Robert J.; Bishop, David F.; Ioannou, Yiannis A.
- TI Cloning and expression of biologically active  $\alpha\text{-}$  galactosidase A
- SO U.S., 73 pp. Cont.-in-part of U.S. Ser. No. 602,824. CODEN: USXXAM

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	US	5356	804			Α		1994	1018	•	US 1:	990-	6028	324		19	9901	024	
	US	5382	524			Α		1995	0117		US 1:	990-	6026	808		19	9901	024	
	EΡ	1375	665			A1		2004	0102		EP 2	003-	1106	51		19	9911	023	
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	WO	9412	628			A1		1994	0609	1	WO 1:	993-	US11	1539		19	9931	130	
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- AU Schmidt B; Selmer T; Ingendoh A; von Figura K
- TI A novel amino acid modification in sulfatases that is defective in multiple sulfatase deficiency.
- SO Cell, (1995 Jul 28) Vol. 82, No. 2, pp. 271-8. Journal code: 0413066. ISSN: 0092-8674.
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		MW,	NO,	NZ,	PL,	RO,	RU,	SD,	SK,	UΑ,	UZ							
	RW:	AT,	BE,	CH,	DE,	DK,	ES,	FR,	GB,	GR,	ΙE,	IT,	LU,	MC,	NL,	PT,	SE,	
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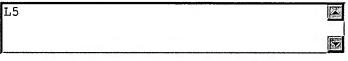
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<u>L2</u>	lysosomal near4 hydrolase or galactosidase or sulfatase or neuraminidase or ceramidase or glucosaminidase or glucosidase or glucosidase	59188	<u>L2</u>
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